Disease Assessment at Diagnosis

1. What was the date of diagnosis of Wiskott-Aldrich Syndrome (WAS)?

2. Specify the WAS defining (diagnostic) criteria:
   1. definitive
      (definitive diagnosis defined as male patient with congenital thrombocytopenia (<70,000 platelets/mm³), small platelets, and at least one of the additional criteria at questions 3–6)
   2. probable
      (probable diagnosis defined as male patient with congenital thrombocytopenia (<70,000 platelets/mm³), small platelets, and at least one of the additional criteria at questions 7–10)
   3. possible
      (possible diagnosis defined as male patient with congenital thrombocytopenia (<70,000 platelets/mm³) and small platelets; or with splenectomy for thrombocytopenia and at least one of the additional criteria at questions 7–10)

Specify all additional criteria for definitive WAS diagnosis:

3. 1 yes 2 no Mutation in WASp
4. 1 yes 2 no Absent WASp mRNA on northern blot analysis of lymphocytes
5. 1 yes 2 no Absent WASp protein in lymphocytes
6. 1 yes 2 no Maternal cousins, uncles or nephews with small platelets and thrombocytopenia

Specify all additional criteria for probable / possible WAS diagnosis:

7. 1 yes 2 no Eczema
8. 1 yes 2 no Abnormal antibody response to polysaccharide antigens
9. 1 yes 2 no Autoimmune disease(s)
10. 1 yes 2 no Lymphoma / leukemia
11. Was a WAS gene mutation identified?
1 □ yes 2 □ no

12. Specify gene mutation identified:
1 □ nucleotides affected (e.g., 361C>T)
2 □ predicted amino acid change (e.g., W14R)

13. Was a WASp protein expressed?
1 □ yes 2 □ no 3 □ unknown

Laboratory Studies at Diagnosis
Report findings prior to any first treatment of Wiskott-Aldrich syndrome.

14. Date CBC tested: (testing done within 6 weeks of diagnosis)

15. WBC: □ not tested
   1 □ x 10^9/L (x 10^3/mm^3)
   2 □ x 10^6/L

16. Lymphocytes: □ % □ not tested

17. Eosinophils: □ % □ not tested

18. Polymorphonuclear leukocytes (PMN): □ % □ not tested

19. Hemoglobin: □ g/dL □ g/L □ mmol/L
   1 □ g/dL □ g/L □ mmol/L □ not tested □ transfused RBC < 30 days from date of test

20. Platelets: □ x 10^9/L (x 10^3/mm^3) □ not tested □ transfused platelets < 7 days from date of test

21. Mean platelet volume: □ fl □ not tested

Immunoglobulin Analysis
Specify the following quantitative immunoglobulins measured prior to any disease treatment:

Value: Specify units:

22. IgG: □ mg/dL □ g/dL □ g/L
   1 □ mg/dL □ g/dL □ g/L □ not tested

24. IgM: □ mg/dL □ g/dL □ g/L
   1 □ mg/dL □ g/dL □ g/L □ not tested

26. IgA: □ mg/dL □ g/dL □ g/L
   1 □ mg/dL □ g/dL □ g/L □ not tested

28. IgE: □ IU/mL □ not tested

30. Did the recipient receive supplemental intravenous immunoglobulins (IVIG) prior to any first treatment of WAS?
1 □ yes 2 □ no 3 □ unknown

31. Was therapy ongoing within one month of immunoglobulin testing?
1 □ yes 2 □ no
**CIBMTR Recipient ID:**

**CIBMTR Center Number:**

**Today's Date:**

20 0

**Infusion Date:**

20 0

**CIBMTR Center Number:**

**CIBMTR Recipient ID:**

---

**Lymphocyte Analysis**

Specify the following lymphocyte analyses performed prior to any disease treatment:

32. Were lymphocyte analyses performed? [ ]

   1. Yes
   2. No

33. Date of most recent testing performed:

Month Day Year

34. Absolute lymphocyte count: 

   - or - 

   cells / µL (cells / mm³)

   Value: 

35. CD3 (T cells):

   - or - 

   % of total lymphocytes:

   Value: 

   Specify units:

   1. x 10⁹/L (x 10³/mm³)
   2. x 10⁶/L

36. CD4 (T helper cells):

   - or - 

   % of total lymphocytes:

   Value: 

   Specify units:

   1. x 10⁹/L (x 10³/mm³)
   2. x 10⁶/L

37. CD8 (cytotoxic T cells):

   - or - 

   % of total lymphocytes:

   Value: 

   Specify units:

   1. x 10⁹/L (x 10³/mm³)
   2. x 10⁶/L

38. CD20 (B lymphocyte cells):

   - or - 

   % of total lymphocytes:

   Value: 

   Specify units:

   1. x 10⁹/L (x 10³/mm³)
   2. x 10⁶/L

39. CD56 (natural killer (NK) cells):

   - or - 

   % of total lymphocytes:

   Value: 

   Specify units:

   1. x 10⁹/L (x 10³/mm³)
   2. x 10⁶/L

40. CD4+ / CD45RA+ (naive T cells):

   - or - 

   % of total lymphocytes:

   Value: 

   Specify units:

   1. x 10⁹/L (x 10³/mm³)
   2. x 10⁶/L

41. CD4+ / CD45RO+ (memory T cells):

   - or - 

   % of total lymphocytes:

   Value: 

   Specify units:

   1. x 10⁹/L (x 10³/mm³)
   2. x 10⁶/L

---

**Clinical Features Assessed between Diagnosis and the Start of the Preparative Regimen**

**Infections Identified between Diagnosis and the Start of the Preparative Regimen**

Specify the presence of all clinically significant infections identified between diagnosis and the start of the preparative regimen. If any given infection was identified, use the Codes for Commonly Reported Organisms on the following page to report the organism present. Only report an organism once, even if it was identified at the same site in subsequent infections. For questions 66–78, also report any fungal infections in the Form 2000 – Recipient Baseline Data beginning at question 163.

- **Copy this chart to report more than three different infections identified at any one site; check here [ ] if additional pages are attached.**

**Site of infection?**

42. 1. Yes 2. No  

   - Hepatitis

   43. First organism

   44. Second organism

   45. Third organism

   46. Specify other organism

47. If hepatitis was present, was it a prominent feature of WAS?  

   1. Yes  

   2. No

48. 1. Yes 2. No  

   - Meningitis / encephalitis

   49. First organism

   50. Second organism

   51. Third organism

   52. Specify other organism

---

Fax this form to your designated campus (Milwaukee 414-805-0714 or Minneapolis 612-627-5895).
ERROR CORRECTION FORM

Sequence Number: ____________________  CIBMTR Recipient ID: ____________________  Initials: ____________________

Today's Date: ____________________  Infusion Date: ____________________  CIBMTR Center Number: ____________________

Month  Day  Year  Month  Day  Year

CIBMTR Center Number: ____________________  CIBMTR Recipient ID: ____________________

Site of infection?

<table>
<thead>
<tr>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
<th>8</th>
<th>9</th>
<th>10</th>
<th>11</th>
<th>12</th>
<th>13</th>
<th>14</th>
<th>15</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pneumonia</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
| | yes | 2 | no
59. If pneumonia was present, was it a prominent feature of WAS?
<table>
<thead>
<tr>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
<th>8</th>
<th>9</th>
<th>10</th>
<th>11</th>
<th>12</th>
<th>13</th>
<th>14</th>
</tr>
</thead>
</table>
| | yes | 2 | no
60. If severe or protracted diarrhea was present, was it a prominent feature of WAS?
<table>
<thead>
<tr>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
<th>8</th>
<th>9</th>
<th>10</th>
<th>11</th>
<th>12</th>
</tr>
</thead>
</table>
| | yes | 2 | no
65. If diarrhea was present, was it a prominent feature of WAS?
<table>
<thead>
<tr>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
<th>8</th>
<th>9</th>
<th>10</th>
</tr>
</thead>
</table>
| | yes | 2 | no
66. If systemic infection was present, was it a prominent feature of WAS?
<table>
<thead>
<tr>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
<th>8</th>
<th>9</th>
</tr>
</thead>
</table>
| | yes | 2 | no
67. If systemic infection other than pneumonia was present, was it a prominent feature of WAS?
<table>
<thead>
<tr>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
<th>8</th>
</tr>
</thead>
</table>
| | yes | 2 | no
71. If systemic infection was present, was it a prominent feature of WAS?
<table>
<thead>
<tr>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
<th>8</th>
<th>9</th>
<th>10</th>
</tr>
</thead>
</table>
| | yes | 2 | no
72. If other infection was present, was it a prominent feature of WAS?
<table>
<thead>
<tr>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
<th>8</th>
<th>9</th>
</tr>
</thead>
</table>
| | yes | 2 | no
77. Specify other infection site:
<table>
<thead>
<tr>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7</th>
<th>8</th>
<th>9</th>
<th>10</th>
<th>11</th>
<th>12</th>
<th>13</th>
<th>14</th>
<th>15</th>
</tr>
</thead>
<tbody>
<tr>
<td>Other infection</td>
<td>73</td>
<td>74</td>
<td>75</td>
<td>76</td>
<td>77</td>
<td>78</td>
<td>79</td>
<td>80</td>
<td>81</td>
<td>82</td>
<td>83</td>
<td>84</td>
<td>85</td>
<td>86</td>
</tr>
</tbody>
</table>
| | yes | 2 | no

Codes for Commonly Reported Organisms

<table>
<thead>
<tr>
<th>#</th>
<th>Organism</th>
</tr>
</thead>
<tbody>
<tr>
<td>104</td>
<td>Listeria</td>
</tr>
<tr>
<td>150</td>
<td>Methylobacterium</td>
</tr>
<tr>
<td>151</td>
<td>Micrococcus, NOS</td>
</tr>
<tr>
<td>112</td>
<td>Mycobacterium avium complex (MAC, M. avium-intracellularium)</td>
</tr>
<tr>
<td>174</td>
<td>Nocardia asteroides</td>
</tr>
<tr>
<td>100</td>
<td>Nocardia brasiliensis</td>
</tr>
<tr>
<td>196</td>
<td>Nocardia farcinica</td>
</tr>
<tr>
<td>147</td>
<td>Lactobacillus (bulgaricus, acidophilus, other species)</td>
</tr>
<tr>
<td>146</td>
<td>Klebsiella</td>
</tr>
<tr>
<td>145</td>
<td>Helicobacter pylori</td>
</tr>
<tr>
<td>139</td>
<td>Fusobacterium</td>
</tr>
<tr>
<td>138</td>
<td>Flavobacterium</td>
</tr>
<tr>
<td>137</td>
<td>Flavimonas oryzihabitans</td>
</tr>
</tbody>
</table>

Fax this form to your designated campus (Milwaukee 414-805-0714 or Minneapolis 612-627-5895).
Clinical Status Between Diagnosis and the Preparative Regimen

79. Did the recipient undergo a splenectomy (between diagnosis and prior to the preparative regimen)?

1. yes
2. no
3. unknown

80. Specify the date the splenectomy was performed: [Month Day Year]

81. Platelets (after splenectomy):
   1. < 10^9/L
   2. < 10^6/L
   3. not tested
   4. transfused platelets < 7 days from date of test

82. Were thrombocytopenia (< 100 x 10^9/L) and small platelets present without any other symptoms, clinical findings, or laboratory abnormalities attributable to WAS (between diagnosis and prior to the preparative regimen)?

1. yes
2. no
3. unknown

Specify thrombocytopenia in the Form 2000 — Recipient Baseline Data at questions 117–118

83. Was eczema present as a clinical feature (between diagnosis and prior to the preparative regimen)?

1. yes
2. no
3. unknown

Specify severity of eczema:
   1. mild, transient
   2. persistent but manageable
   3. difficult to control

85. Was a coexisting malignancy present (between diagnosis and prior to the preparative regimen)?

1. yes
2. no
3. unknown

Specify malignancy:

Report malignancy in the Form 2000 — Recipient Baseline Data at questions 23–60

87. Did the recipient experience any of the following types of bleeding episodes (between diagnosis and prior to the preparative regimen)?

1. yes
2. no

Specify types of bleeding:

<table>
<thead>
<tr>
<th>Bleeding episode(s) present?</th>
<th>If present, is the feature prominent?</th>
</tr>
</thead>
<tbody>
<tr>
<td>Epistaxis</td>
<td></td>
</tr>
<tr>
<td>Upper GI hemorrhage</td>
<td></td>
</tr>
<tr>
<td>Lower GI hemorrhage</td>
<td></td>
</tr>
<tr>
<td>Rectal bleeding</td>
<td></td>
</tr>
<tr>
<td>Hemorrhagic bowel bleeding</td>
<td></td>
</tr>
<tr>
<td>Oral bleeding</td>
<td></td>
</tr>
<tr>
<td>Subcutaneous bleeding</td>
<td></td>
</tr>
<tr>
<td>Subdural hematoma</td>
<td></td>
</tr>
<tr>
<td>Other bleeding</td>
<td></td>
</tr>
</tbody>
</table>

Report GI hemorrhage in the Form 2000 — Recipient Baseline Data at question 63
Report GU hemorrhage in the Form 2000 — Recipient Baseline Data at question 64
Report CNS hemorrhage in the Form 2000 — Recipient Baseline Data at question 65

Fax this form to your designated campus (Milwaukee 414-805-0714 or Minneapolis 612-627-5895).
109. Did the recipient experience any of the following autoimmune / inflammatory disorders (between diagnosis and prior to the preparative regimen?)

1 o yes 2 o no

Specify autoimmune / inflammatory disorders:

110. 1 o yes 2 o no Arthralgia
111. 1 o yes 2 o no Arthritis, chronic
112. 1 o yes 2 o no Autoimmune hemolytic anemia
113. 1 o yes 2 o no Autoimmune thrombocytopenic purpura (ITP)
114. 1 o yes 2 o no Inflammatory bowel disease
115. 1 o yes 2 o no Juvenile rheumatoid arthritis
116. 1 o yes 2 o no Nephritis
117. 1 o yes 2 o no Neutropenia
118. 1 o yes 2 o no Vasculitis, cerebral
119. 1 o yes 2 o no Vasculitis, coronary
120. 1 o yes 2 o no Vasculitis, renal
121. 1 o yes 2 o no Vasculitis, skin
122. 1 o yes 2 o no Vasculitis, other
123. 1 o yes 2 o no Sclerosing cholangitis
124. 1 o yes 2 o no Vasculitis, other
125. 1 o yes 2 o no Vasculitis, skin
126. 1 o yes 2 o no Vasculitis, other
127. 1 o yes 2 o no Vasculitis, other
128. 1 o yes 2 o no Vasculitis, other
129. 1 o yes 2 o no Vasculitis, other
130. 1 o yes 2 o no Vasculitis, other
131. 1 o yes 2 o no Vasculitis, other
132. 1 o yes 2 o no Vasculitis, other
133. 1 o yes 2 o no Vasculitis, other
134. 1 o yes 2 o no Vasculitis, other
135. 1 o yes 2 o no Vasculitis, other
136. 1 o yes 2 o no Vasculitis, other
137. 1 o yes 2 o no Vasculitis, other
138. Specify other vasculitis:
139. 1 o yes 2 o no Other disorder
140. 1 o yes 2 o no Other disorder

141. Specify other disorder:

If present, is the feature prominent?

142. Were any biologic specimens collected for this recipient (between the date of diagnosis and the preparative regimen)?

1 o yes 2 o no 3 o unknown

Specify if specimen(s) collected and available for future research:

143. 1 o yes 2 o no DNA
144. 1 o yes 2 o no Epstein-Barr virus (EBV)-transformed B-cell line
145. 1 o yes 2 o no Fibroblast cell line
146. 1 o yes 2 o no Herpes virus saimiri-transformed T-cell line
147. 1 o yes 2 o no Other T-cell line
148. 1 o yes 2 o no Pathological specimen
149. Specify pathological specimen(s):
150. 1 o yes 2 o no Peripheral blood mononuclear cells (PBMC), frozen
151. 1 o yes 2 o no RNA
152. Specify RNA source:
153. 1 o yes 2 o no Serum (pre-IVIG)
154. 1 o yes 2 o no Other specimen
155. Specify other specimen(s):

Fax this form to your designated campus (Milwaukee 414-805-0714 or Minneapolis 612-627-5895).